

#4

<b>LIST OF REFERENCES CITED BY APPLICANT</b> (Use several sheets if necessary) <b>PTO FORM 1449</b>		ATTY. DOCKET NO.	APPLICATION NO.
		00800.0051.CNUS03	09/993,241
		APPLICANT	
		Emil Kakkis	
		FILING DATE	GROUP
		November 13, 2001	1652



RECEIVED  
 FEB 14 2002  
 TECH CENTER 1600/2900

U.S. PATENT DOCUMENTS						
*EXAMINER INITIAL		DOCUMENT NUMBER	DATE	NAME	CLASS	SUBCLASS
*mm2		3,472,931	10/1969	Stoughton	—	—
*		3,891,757	06/1975	Higuchi	—	—
*mm2		5,270,051	12/1993	Harris	—	—

FOREIGN PATENT DOCUMENTS								
*EXAMINER INITIAL		DOCUMENT NUMBER	DATE	COUNTRY	CLASS	SUBCLASS	TRANSLATION	
							YES	NO
*mm2		WO 93/10244	05/1993	PCT	—	—		
*↓		1,001,949	08/1965	United Kingdom	—	—		
↓	✓	WO 99/58691	11/18/99	PCT	—	—	X	
mm2	✓	WO 99/51724	10/14/99	PCT	—	—	X	

OTHER REFERENCES	
(Including Author, Title, Date, Pertinent Pages, Etc.)	
*mm2	Anson, D. S., et al., "Correction of Human Mucopolysaccharidosis Type -VI Fibroblasts with Recombinant N-Acetylgalactosamine-4-Sulphatase," <i>Biochem J.</i> - 284:789-794 (1992)
*	Barton, R. W., et al., "The Hurler Corrective Factor," <i>J. Biol. Chem.</i> - 246(24):7773-7779 (1971)
*	Bielicki, J., et al., "Recombinant Human Iduronate-2-Sulphatase: Correction of Mucopolysaccharidosis-Type II Fibroblasts and Characterization of the Purified Enzyme," <i>Biochem. J.</i> - 289:241-246 (1993)
✓	Clements, et al., "Human alpha-L-iduronidase 1. Purification, monoclonal antibody production, native and subunit molecular mass," <i>European Journal of Biochemistry</i> , 152(1):43-49 (1994)
*	Friedman, T., "Progress Toward Human Gene Therapy," <i>Science</i> - 244:1275-1281 (1989)
*	Hoogerbrugge, P.M., et al., "Allogeneic Bone Marrow Transplantation for Lysosomal Storage Diseases," <i>Lancet</i> - 345:1398 (1995)
*	Ioannou, Y.A., et al., "Overexpression of Human $\alpha$ -Galactosidase A Results in Its Intracellular Aggregation, Crystallization in Lysosomes, and Selective Secretion," <i>J. Cell Biol.</i> - 119(5):1137-1150 (1992)
mm2	✓ Kakkis, et al., "Enzyme-replacement therapy in mucopolysaccharidosis I.," <i>New England Journal of Medicine</i> , 344(3):182-188 (2001)

\*EXAMINER: Initial if reference considered, whether or not citation is in conformance with MPEP 609; Draw line through citation if not in conformance and not considered. Include copy of this form with next communication to applicant.

<b>LIST OF REFERENCES CITED BY APPLICANT</b> (Use several sheets if necessary) <b>PTO FORM 1449</b>		ATTY. DOCKET NO. 00800.0051.CNUS03	APPLICATION NO. 09/993,241
		APPLICANT Emil Kakkis	
		FILING DATE November 13, 2001	GROUP 1652



* <i>mm</i>		Kakkis, E., et al., "Strong Transcriptional Activation of Translocated C-Myc Genes Occurs Without a Strong Nearby Enhancer or Promoter," <i>Nucleic Acids Res.</i> - <u>16</u> (1):77-96 (1988)
*		Ledley, F.D., "Clinical Application of Somatic Gene Therapy in Inborn Errors of Metabolism," <i>Inherit. Metab. Dis.</i> - <u>13</u> :597-616 (1990)
*		Lowry, R.B., et al. "An Update on the Frequency of Mucopolysaccharide Syndromes in British Columbia," <i>Human Genetics</i> - <u>85</u> :389-390 (1990)
*		Myerowitz, R., et al., "Maturation of $\alpha$ -L-Iduronidase in Cultured Human Fibroblasts," <i>J. Biol. Chem.</i> - <u>256</u> (6):3044-3048 (1981)
*		Moskowitz, S.M., et al., "Cloning and Expression of cDNA Encoding the Human Lysosomal Enzyme, $\alpha$ -L-Iduronidase," <i>FASEB J.</i> - <u>6</u> :A77 (1992)
*		Nelson, J., "Incidence of the Mucopolysaccharidoses in Northern Ireland," <i>Human Genetics</i> - <u>101</u> :355-358 (1997)
*		Scriver, C.R., Beaudet, A.L., Sly, W.S. and Valle, D. Eds. <u>The Metabolic Basis of Inherited Disease</u> pp 1565-1587, McGraw Hill, New York (1989)
*		Shull, R.M., et al., "Enzyme Replacement in a Canine Model of Hurler Syndrome," <i>Proc. Natl. Acad. Sci., USA</i> - <u>91</u> :12937-12941 (1994)
*		Stoltzfus, L.J., et al., "Cloning and Characterization of cDNA Encoding Canine $\alpha$ -L-Iduronidase," <i>J. Biol. Chem.</i> - <u>267</u> (10):6570-6575 (1992)
*		Taylor, J., et al., " $\alpha$ -L-Iduronidase in Normal and Mucopolysaccharidosis -Type-1 Human Skin Fibroblasts," <i>Biochem J.</i> - <u>274</u> :263-268 (1991)
*		Tolstoshev, P., et al., "Gene Expression Using Retroviral Vectors," <i>Current Opinions Biotech.</i> - <u>1</u> :55-61 (1990)
*		Tucker, P.W., et al., "Mouse IgA Heavy Chain Gene Sequence: Implications for Evolution of Immunoglobulin Hinge Exons," <i>Proc. Natl. Acad. Sci. USA</i> - <u>78</u> (12):7684-7688 (1981)
* <i>mm</i>		Unger, E.G., et al., "Recombinant $\alpha$ -L-Iduronidase: Characterization of the Purified Enzyme and Correction of Mucopolysaccharidosis Type I Fibroblasts," <i>Biochem. J.</i> - <u>304</u> :43-49 (1994)

\*EXAMINER: Initial if reference considered, whether or not citation is in conformance with MPEP 609; Draw line through citation if not in conformance and not considered. Include copy of this form with next communication to applicant.

RECEIVED

FEB 14 2002  
TECH CENTER 1600/2000

<b>LIST OF REFERENCES CITED BY APPLICANT</b> (Use several sheets if necessary)  <b>PTO FORM 1449</b>		ATTY. DCK 00800.0051.CNUS03	APPLICATION NO. 09/993,241
		APPLICANT Emil D. KAKKIS	
		FILING DATE November 13, 2001	GROUP 1652

U.S. PATENT DOCUMENTS							
*EXAMINER INITIAL	DOCUMENT NUMBER	DATE	NAME	CLASS	SUBCLASS	FILING DATE IF APPROPRIATE	
mmr	6,149,909	11/21/2000	Scott, <i>et al.</i>	424	94.61	11/13/1998	
	6,238,662 B1	5/29/2001	Scott, <i>et al.</i>	424	94.61	8/30/1999	

  

FOREIGN PATENT DOCUMENTS							
*EXAMINER INITIAL	DOCUMENT NUMBER	DATE	COUNTRY	CLASS	SUBCLASS	TRANSLATION	
						YES	NO
mmr	WO 97/10353	3/20/1997	PCT				

  

OTHER REFERENCES	
(Including Author, Title, Date, Pertinent Pages, Etc.)	
mmr	Kakkis, <i>et al.</i> , "Long-Term and High-Dose Trials of Enzyme Replacement Therapy in the Canine Model of Mucopolysaccharidosis I", <i>Biochem. Mol. Med.</i> Vol. 58, No. 2, pp. 156-167 (1996).
	Neufeld, <i>et al.</i> , "The Mucopolysaccharidoses", <u>The Metabolic Basis of Inherited Disease</u> , Scriver, C.R., Beaudet, A. L., Sly, W.S., and Valle, D. Eds. McGraw Hill, New York, pp. 1565-1587 (1989).
	Rome, <i>et al.</i> , "a-L-Iduronidase for Human Kidney" <i>Methods in Enzymology</i> , Vol. 83, pp. 578-582 (1982).
	Schuchman, <i>et al.</i> , "Human a-L-Iduronidase: Purification and Properties of the High Uptake (Higher Molecular Weight) and the Low Uptake (Processed) Forms" <i>J. Bio. Chem.</i> , Vol. 259, No. 5, pp. 3132-3140 (1984).
	Scott, <i>et al.</i> , "Human a-L-Iduronidase: cDNA Isolation and Expression", <i>Proc. Natl. Acad. Sci. USA</i> Vol. 88, pp. 9695-9699, (1991).
	Scott, <i>et al.</i> , "Multiple Polymorphisms Within the a-L-Iduronidase Gene ( <i>IDUA</i> ): Implications for a Role in Modification of MPS-I Disease Phenotype", <i>Hum. Mol. Genet.</i> Vol. 2, No. 9, pp. 1471-1473 (1993).
mmr	Stoltzfus, <i>et al.</i> , "Mucopolysaccharidosis I: cloning and characterization of cDNA encoding canine a-L-Iduronidase", <i>Am. J. Hum. Genet.</i> , Vol. 47, No. 3, p. A167, (1990).

\*EXAMINER: Initial if reference considered, whether or not citation is in conformance with MPEP 609; Draw line through citation if not in conformance and not considered. Include copy of this form with next communication to applicant.

# LIST OF REFERENCES CITED BY APPLICANT

(Use several sheets if necessary)

PTO FORM 1449

ATTY. DOCKET NO.

00800.0051.CNUS03

APPLICATION NO.

09/993,241

APPLICANT

Emil D. KAKKIS

FILING DATE

November 13, 2001

GROUP

1652

## U.S. PATENT DOCUMENTS

*EXAMINER INITIAL	DOCUMENT NUMBER	DATE	NAME	CLASS	SUBCLASS	FILING DATE IF APPROPRIATE

## FOREIGN PATENT DOCUMENTS

*EXAMINER INITIAL	DOCUMENT NUMBER	DATE	COUNTRY	CLASS	SUBCLASS	TRANSLATION
						YES NO

## OTHER REFERENCES

(Including Author, Title, Date, Pertinent Pages, Etc.)

mm2		Clements, <i>et al.</i> , "Immunopurification and characterization of human $\alpha$ -L-iduronidase with the use of monoclonal antibodies", <i>Biochem. J.</i> Vol. 259, pp. 199-208 (1989).
		Kakkis, <i>et al.</i> , "Overexpression of the human lysosomal enzyme $\alpha$ -L-iduronidase in Chinese Hamster Ovary cells", <i>Prot. Exp. Purif.</i> Vol. 5, pp. 225-232 (1994).
		Scott, <i>et al.</i> , "Chromosomal localization of the human $\alpha$ -L-iduronidase gene (IDUA) to 4p16.3" <i>Am. J. Hum. Genet.</i> Vol. 47, pp. 802-807 (1990).
mm2		Zhao, <i>et al.</i> , "Carbohydrate structures of recombinant human $\alpha$ -L-iduronidase secreted by Chinese Hamster Ovary cells", <i>J. Biol. Chem.</i> Vol. 273, No. 36, pp. 22758-22765 (1997).

\*EXAMINER: Initial if reference considered, whether or not citation is in conformance with MPEP 609; Draw line through citation if not in conformance and not considered. Include copy of this form with next communication to applicant.

**LIST OF REFERENCES CITED BY APPLICANT**

(Use several sheets if necessary)

**PTO FORM 1449**

ATTY. DOC

00800.0051.CNUS03

APPLICATION NO.

09/993,241

#11

APPLICANT

Emil D. KAKKIS

FILING DATE

November 13, 2001

GROUP

1652

**U.S. PATENT DOCUMENTS**

*EXAMINER INITIAL	DOCUMENT NUMBER	DATE	NAME	CLASS	SUBCLASS	FILING DATE IF APPROPRIATE

**FOREIGN PATENT DOCUMENTS**

*EXAMINER INITIAL	DOCUMENT NUMBER	DATE	COUNTRY	CLASS	SUBCLASS	TRANSLATION	
						YES	NO
	EP 1 029 548 A	8/23/02	United Kingdom				

**OTHER REFERENCES**

(Including Author, Title, Date, Pertinent Pages, Etc.)

mm	"BioMarin and Genzyme Report positive One Year Summary Data For MPS-1 Patients" <del>GENZYME GENERAL WEB SITE: WWW.GENZYME.COM, Online!</del> 14 September 1999 (1999-09-14), Retrieved from the Internet: <URL: <a href="http://www.prnewswire.com/cgi-bin/stories.pl?acct=105&amp;story=/www/story/09-14-1999/0001020970">http://www.prnewswire.com/cgi-bin/stories.pl?acct=105&amp;story=/www/story/09-14-1999/0001020970</a> > retrieved on 2002-08-08
----	--

**RECEIVED**  
DEC 11 2002  
TECH CENTER 1600/2900

\*EXAMINER: Initial if reference considered, whether or not citation is in conformance with **MPEP 609**; Draw line through citation if not in conformance and not considered. Include copy of this form with next communication to applicant.